Unleashing the cure: Overcoming persistent obstacles in the translation and expanded use of hematopoietic stem cell-based therapies.

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Public Summary:
Hematopoietic stem cell transplantation (HSCT) is broadly used for treating and curing hematological cancers and various disorders of the blood and immune system. However, its true therapeutic potential remains vastly constrained by significant scientific and technical hurdles that preclude expansion to new indications and limit the number of patients who could benefit from, gain access to, or financially afford the procedure. To define and overcome these challenges, the California Institute for Regenerative Medicine (CIRM) held multiple workshops related to HSCT and has subsequently invested in a new generation of approaches to address the most compelling needs of the field, including new sources of healthy and immunologically compatible hematopoietic stem cells for transplant; safe and efficient genome modification technologies for correction of inherited genetic defects and other forms of gene therapy; safer and more tractable transplantation procedures such as nongenotoxic conditioning regimens, methods to accelerate immune reconstitution and recovery of immune function, and innovations to minimize the risk of immune rejection; and other life-threatening complications from transplant. This Perspective serves to highlight these needs through examples from the recent CIRM-funded and other notable investigations, presents rationale for comprehensive, systematic, and focused strategies to unleash the full potential of HSCT, thereby enabling cures for a greatly expanded number of disorders and making HSCT feasible, accessible, and affordable to all who could benefit.